

[Return to CIRM Meetings and Events](#)

## Brainstorming Neurodegeneration



# BRAINSTORMING NEURODEGENERATION

*Leveraging Genomics, Stem Cells, Gene  
Therapy and Novel Clinical Trials  
for Field-wide Advancement*

### About

In April 2019, CIRM hosted a pan-neurodegeneration workshop meeting bringing together ~50 key opinion leaders from a diverse global cross section of regenerative medicine, stem cell research and development, disease foundations, academia, industry, funding bodies and the FDA. The main goal of this meeting was to enable a free-flowing discussion or a "brainstorming session" where the attendees would tackle issues experienced in the development of neurodegenerative therapies by applying current state of the art techniques and platforms to reconfigure discovery and development pipelines and finding synergies with groups/consortia. The meeting was held over 2 days and covered topics ranging from leveraging genomics data and patient derived induced pluripotent stem cell models for discovery research to a discussion of clinical development strategies and regulatory paradigms specific to a new generation of therapies.

The breakdown of specific goals discussed as well as individual sessions of the workshop are presented below:

### Meeting Goals

- Discuss novel models to accelerate therapeutic development for neurodegenerative diseases (NDs)
- Discuss proof of concept examples where genomics and large datasets have enabled progress in ND
- Prioritize elements of common utility
- Explore benefits and considerations for a neutral collective effort across NDs
- Discuss incentive structures to encourage alignment
- Propose an operational framework(s) to move from concept to reality

## Workshop Sessions

- Session I: Leveraging Genomics and Big Data
- Session II: iPSC Models, Creating Standards, Utilizing Banks
- Session III: Exploring a Neurodegeneration Consortium Model
- Session IV: Accelerating Drug Development Based on Patient Data
- Session V: Clinical Trials in Regenerative Medicine – Benefits of a Consortium
- Session VI: Taking Regenerative Medicine ND Candidates to the Clinic
- Breakout Session: Assessing the Value of a Neurodegeneration
- Session VII: Would an ND Consortium Accelerate Therapy Development: Considerations for a Path Forward

## Materials

Download materials from the workshop using the links below:

- Brainstorming Neurodegeneration Agenda
- Meeting Summary Document

## Presentation Slides

Download the presentation slides from the workshop using the links below:

- Session Overview Slides
- Introduction
- Meeting Objectives
- Plenary – Regulatory Pathway
- Plenary – Accelerating CNS
- Session I
- Session IIA
- Session IIB
- Session III
- Session IV
- Session V
- Plenary – Federal Funding
- Session VI

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